Beam Therapeutics Names First CAR-T Base Editing Development Candidate for the Treatment of T-ALL and Presents New Data at SITC 2020

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BEAM-201, an Off the Shelf Allogeneic CD7-Targeting CAR-T, Named as Development Candidate for Treatment of T-Cell Acute Lymphoblastic Leukemia

First Cell Therapy Featuring Simultaneous Edits to Four Genes; Demonstrates 96-99% On-target Editing at Clinical Scale and In Vivo Proof of Concept of Tumor Clearance

CAMBRIDGE, Mass., Nov. 09, 2020 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced that the company will advance BEAM-201, a potent and specific anti-CD7, multiplex edited, allogeneic CAR-T therapy, as a development candidate for the treatment of T-cell acute lymphoblastic leukemia (T-ALL). Preclinical data on BEAM-201 are being presented in a poster session during the Society for Immunotherapy of Cancer's 35th Anniversary Annual Meeting & Pre-Conference Programs (SITC 2020), and demonstrate potent, dose-dependent tumor control *in vitro* and in an *in vivo* xenograft model.

"BEAM-201 is the third development candidate from our pipeline of base editing programs named this year, an incredible milestone for our company and a testament to the strength of our platform and the dedication of our team," said Giuseppe Ciaramella, Ph.D., president and chief scientific officer of Beam. "BEAM-201 is a highly differentiated editing program designed to provide an 'off-the-shelf' CD7-targeting CAR-T cell therapy that may enable a one-time treatment option for patients with T-ALL. To our knowledge, BEAM-201 is the first cell therapy featuring simultaneous edits to four genes. The preclinical data being presented at SITC 2020 demonstrate 96-99% editing efficiencies across four targets without genomic rearrangements, as well as strong *in vivo* proof of concept of tumor clearance in a xenograft model. We are actively advancing BEAM-201 to assess its potential impact in treating people living with this devastating disease."

BEAM-201 is a potent and specific anti-CD7, multiplex edited, allogeneic CAR-T development candidate for the treatment of relapsed/refractory T-ALL, a severe disease affecting children and adults with a five-year overall survival of less than 25%. BEAM-201 is produced using a GMP-compliant, clinical-scale process in which T cells derived from healthy donors are simultaneously base edited at four genomic loci then transduced with a lentivirus coding for an anti-CD7 CAR. The resulting cells are universally-compatible, allogeneic ("off the shelf") CD7-targeting CAR-T cells resistant to both fratricide and immunosuppression.

Details of Beam's SITC 2020 presentation of BEAM-201 are as follows:

Title: Highly efficient multiplexed base editing enables the development of investigational universal CD7-targeting CAR-T Cells to treat T-ALL
Publication Number: 111
Category: Cellular Therapies
Data Summary:

- *In vitro* characterization of the effects of base editing in BEAM-201 demonstrated:
  - Simultaneous base editing by a cytosine base editor (CBE) at four target loci in primary human T cells using a clinical-scale process produced 96-99% on-target editing of each of the four genes as measured by next-generation sequencing and flow cytometry;
  - Simultaneous quad base editing of T cells resulted in no detected genomic rearrangements resulting from the editing process;
  - Multiplex base editing did not negatively affect cell expansion during manufacturing;
  - CBE-edited cells decreased expression of the four target genes with minimal effect on other genes, including key members of the p53 pathway that are upregulated in response to DNA double-stranded breaks produced by multiplex editing with nucleases.
- Further characterization of BEAM-201 *in vitro* and in a tumor mouse model demonstrated:
  - Beam's GMP-compliant, clinical-scale process resulted in final BEAM-201 CAR-T cell populations with on-target editing efficiencies between 96-99.9% at each of the four target loci, and 85% CAR-expressing cells. As a result, Beam estimates that 91% of cells are bi-allelically quad base edited and 77% of cells have all 5 genetic modifications. The company believes this is the highest level and uniformity of CAR expression and simultaneous editing across four target sites reported at clinical scale to date.
  - BEAM-201 cells demonstrated robust *in vitro* CD7-dependent cytokine production, and rapid *in vitro* cytotoxicity.
  - BEAM-201 cells also demonstrated dose-dependent clearance or control, across a 25-fold dose range, of an aggressive disseminated CCRF-CEM T-ALL tumor mouse model.

About Beam Therapeutics
Beam Therapeutics (Nasdaq: BEAM) is a biotechnology company developing precision genetic medicines through the use of base editing. Beam's proprietary base editors create precise, predictable and efficient single base changes, at targeted genomic sequences, without making double-

stranded breaks in the DNA. This enables a wide range of potential therapeutic editing strategies that Beam is using to advance a diversified portfolio of base editing programs. Beam is a values-driven organization committed to its people, cutting-edge science, and a vision of providing life-long cures to patients suffering from serious diseases. For more information, visit www.beamtx.com.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Investors are cautioned not to place undue reliance on these forward-looking statements, including, but not limited to, statements related to: our progress advancing BEAM-201; and the therapeutic applications and potential of our technology, including our ability to develop precision genetic medicines for patients through base editing. Each forward-looking statement is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement, including, without limitation, risks and uncertainties related to: our ability to develop, obtain regulatory approval for, and commercialize our product candidates, which may take longer or cost more than planned; our ability to raise additional funding, which may not be available; our ability to obtain, maintain and enforce patent and other intellectual property protection for our product candidates; the potential impact of the COVID-19 pandemic; that preclinical testing of our product candidates and preliminary or interim data from preclinical and clinical trials may not be predictive of the results or success of ongoing or later clinical trials; that enrollment of our clinical trials may take longer than expected; that our product candidates may experience manufacturing or supply interruptions or failures; risks related to competitive products; and the other risks and uncertainties identified under the heading “Risk Factors” in our Annual Report on Form 10-K for the year ended December 31, 2019, our Quarterly Report on Form 10-Q for the quarters ended March 31, 2020 and June 30, 2020, and in any subsequent filings with the Securities and Exchange Commission. These forward-looking statements (except as otherwise noted) speak only as of the date of this press release. Factors or events that could cause our actual results to differ may emerge from time to time, and it is not possible for us to predict all of them. We undertake no obligation to update any forward-looking statement, whether as a result of new information, future developments or otherwise, except as may be required by applicable law.

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